CIRM-Funded Project Targeting Sickle Cell Disease Gets Green Light for Clinical Trial

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Oakland, CA – The US Food and Drug Administration (FDA) has granted Investigational New Drug (IND) permission enabling Graphite Bio to test the investigational, potentially revolutionary gene editing therapy GPH101 developed under the supervision of Matthew Porteus, MD, PhD, in a clinical trial for people with sickle cell disease (SCD).

The California Institute for Regenerative Medicine (CIRM) has been supporting this project with a $5.2 million grant, enabling Dr. Porteus and his team at the Institute of Stem Cell Biology and Regenerative Medicine at Stanford University to conduct the preclinical manufacturing and safety studies required by the FDA.

“We congratulate the Graphite Bio team for obtaining the IND, a critical step in bringing the GPH101 gene therapy forward for Sickle Cell Disease,” says Dr. Maria T. Millan, CIRM’s President & CEO. “CIRM is committed to the national Cure Sickle Cell initiative and are delighted that this technology, the product of CIRM funded research conducted by Dr. Porteus at Stanford, is progressing to the next stage of development”

Sickle cell disease is caused by a genetic mutation that turns normally smooth, round red blood cells into rigid, sickle shaped cells. Those cells clump together, clogging up blood vessels, causing intense pain, damaging organs and increasing the risk of strokes and premature death. There are treatments that help control the damage, but the only cure is a bone marrow stem cell transplant, which can only happen if the patient has a stem cell donor (usually a close relative) who has matching bone marrow.

The investigational therapy GPH101 harnesses the power of CRISPR and natural DNA repair mechanisms to cut out the single mutation in the sickle globin gene and paste in the correct “code.” Correction of this mutation would reverse the defect and result in healthy non-sickling red blood cells.

CEDAR, a Phase 1/2, multi-center, open-label clinical study is designed to evaluate the safety, preliminary efficacy and pharmacodynamics of GPH101 in adult and adolescent patients with severe SCD.

For patient advocate Nancy Rene, the news is personal: "It's always exciting to hear about the progress being made in sickle cell research. If successful it will mean that my grandson, and especially other young adults, can look forward to a life free of pain and organ damage. They can actually begin to plan their lives, thinking about careers and families. I want to thank Dr. Porteus and all of the scientists who are working so hard for people with sickle cell disease. This is wonderful news."

CIRM has funded four clinical trials for Sickle Cell Disease using different approaches and has a unique partnership with the National Heart, Lung and Blood Institutes under the NIH “Cure Sickle Cell” initiative.

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today’s most promising stem cell technologies.

With $5.5 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world’s largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

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